

Common molecular mechanisms in neurodegenerative diseases using patient based iPSC neurons

Grant Award Details

Common molecular mechanisms in neurodegenerative diseases using patient based iPSC neurons

Grant Type: Basic Biology IV

Grant Number: RB4-06079

Project Objective: Identify common molecular mechanisms of disease in neurodegenerative diseases using neurons and astrocytes derived from iPSC from patients with Huntington's or Parkinson's diseases.

Investigator:

Name:	Steven Finkbeiner
Institution:	Gladstone Institutes, J. David
Type:	PI

Disease Focus: Huntington's Disease, Neurological Disorders, Parkinson's Disease

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: \$1,482,025

Status: Closed

Progress Reports

Reporting Period: Year 1

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Reporting Period: Year 3

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Grant Application Details

Application Title:	Common molecular mechanisms in neurodegenerative diseases using patient based iPSC neurons
Public Abstract:	<p>A major medical problem in CA is the growing population of individuals with neurodegenerative diseases, including Parkinson's (PD) and Huntington's (HD) disease. These diseases affect millions of people, sometimes during the prime of their lives, and lead to total incapacitation and ultimately death. No treatment blocks the progression of neurodegeneration. We propose to conduct fundamental studies to understand the basic common disease mechanisms of neurodegenerative disorders to begin to develop effective treatments for these diseases. Our work will target human stem cells made from cells from patients with HD and PD that are developed into the very cells that degenerate in these diseases, striatal neurons and dopamine neurons, respectively. We will use a highly integrated approach with innovative molecular analysis of gene networks that change the states of proteins in these diseases and state-of-the-art imaging technology to visualize living neurons in a culture dish to assess cause and effect relationships between biochemical changes in the cells and their gradual death. Importantly, we will test whether drugs effective in animal model systems are also effective in blocking the disease mechanisms in the human HD and PD neurons. These human preclinical studies could rapidly lead to clinical testing, since some of the drugs have already been examined extensively in humans in the past for treating other disorders and are safe.</p>
Statement of Benefit to California:	<p>Neurodegenerative diseases, such as Parkinson's (PD) and Huntington's disease (HD), are devastating to patients and families and place a major financial burden on California. No treatments effectively block progression of any neurodegenerative disease. A forward-thinking team effort will allow highly experienced investigators in neurodegenerative disease and stem cell research to investigate common basic mechanisms that cause these diseases. Most important is the translational impact of our studies. We will use neurons and astrocytes derived from patient induced pluripotent stem cells to identify novel targets and discover disease-modifying drugs to block the degenerative process. These can be quickly transitioned to testing in preclinical and clinical trials to treat HD and other neurodegenerative diseases. We are building on an existing strong team of California-based investigators to complete the studies. Future benefits to California citizens include: 1) discovery and development of new HD treatments with application to other diseases, such as PD, that affect thousands of Californians, 2) transfer of new technologies and intellectual property to the public realm with resulting IP revenues to the state with possible creation of new biotechnology spin-off companies, and 3) reductions in extensive care-giving and medical costs. We anticipate the return to the State in terms of revenue, health benefits for its Citizens and job creation will be significant.</p>

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